Need for public-private collaboration

- Novel **Lead Structures** and **Drug Targets** are Innovation Drivers of the Early Small Molecule Drug Discovery Process

- Shift of Pharma discovery portfolio towards challenging (“intractable”) targets

- Limited access of academia to comprehensive compound collections and medicinal chemistry resources

- Combining strengths to enhance early drug discovery process for both, public and private projects
Objectives of the full project

- Provide access to comprehensive, high-quality Joint EU Compound Collection
- Generate “Qualified Hits” for subsequent refinement in drug development process or serving as tool compounds in target research
- Establish novel platform to foster public-private collaboration
- Generate broad knowledge base (Qualified Hits x 240 projects) to directly exploit results or deduce future library design strategies

Joint European Compound Collection

x

Target/Assay

Hit Characterization

Qualified Hit List

Hit-to-Lead Optimization

MedChem Follow-Up

Tool Cpd. for Target Research

up to 500,000 cpds.
Objectives of the full project

The EU Lead Factory

- Generate Comprehensive high-quality Joint European Compound Collection:
  - integrate industry’s medicinal chemistry know-how
  - focus on value (IP) generation => unique, commercially non-available compounds
  - complement “Pharma Collection” by novel Public Compound Collection
    => (i) differentiated chemistry, (ii) targeting ‘intractable targets’

- Establish European Screening Centre as ‘Centre of Excellence’ for
  - housing and managing the Joint EU Compound Collection
  - development of target or pathway-specific bioassays
  - performing HTS for public projects
  - drive experimental hit characterization (selection) process
  - managing data flow and analysis (‘honest broker concept’) and project execution
Objectives of the full project

The EU Lead Factory

- Project realization within time and budget frame requires operational efficiency
- Generation of “Qualified Hits”: medchem properties to match project purpose (Lead or Tool); qualification can include also limited chemistry follow-up activities
- Preference for public projects with relevance to research focus of EFPIA consortium (early partnering) → focus on innovation: No overlap with current or past pharma portfolio
- Information management to balance Intellectual Property (IP) generation with knowledge generation: Disclosure of structural information to project owner (and ultimatively public) limited to Qualified Hit List
Pre-competitive nature

The EU Lead Factory

- Aims to provide industrial lead finding platform to academic programmes
  (i) seeking quality tool compounds for target research
      => validation of novel druggable targets
  (ii) seeking quality compound candidates to enter drug optimization process
      => exploiting also pharma collections outside industry focus for the benefit of patients

- Constitutes ‘controlled experiment’ to broaden scope of pre-competitive research into early stage drug discovery: Collaborative exploitation of compound collections
Expected impact on the R&D process

The EU Lead Factory

- Ideally combines basic and applied research to translate cutting-edge research into early drug discovery programmes

- Provides platform to explore high-risk discovery approaches to share both, success stories and failures

- Provides novel aspect in pre-competitive research: Collaborative exploitation of compound collections in Screening Consortium
Suggested architecture of the project

Applicant Consortium to establish Technology Platforms:
- Screening Centre: Leads or Tools
- Library Synthesis: Public Compound Collection

**EU Lead Factory** provides platforms to collect and translate academia’s cutting-edge science to tangible ‘qualified hits’:

- **Screening Centre:** innovative discovery programs (→ target, pathway, assay) with a clear value proposition
- **Library Synthesis:** innovative library design proposals expanding chemical space through new synthetic methodologies or motifs addressing intractable targets
Expected contributions of the applicants

- Compound Logistics ▪ Assay Development ▪ High-Throughput Screening ▪ Hit Characterization ▪ Medicinal Chemistry Follow-Up ▪ Data Management & Analysis ▪ Project Management

48 HTS Projects *per anno*
- full support for 24 public projects p.a.
- HTS for 24 private projects will be performed by EFPIA Participant

- High-Throughput Chemistry ▪ Compound Library Design ▪ Chemoinformatics ▪ Project Management

- up to 200,000 novel compounds
  (100,000 in the first 3 years)

EoIs should address one of the 2 Sub-Topics; selected applicant groups eventually merge with EFPIA Consortium to generate Full Project Proposal
Expected (in kind) contributions of EFPIA members

1. High quality compounds from in-house collections (from automated and medchem synthesis): 50,000 cpds. → 4 HTS p.a.
2. Execution of private projects (from assay development to HTS) by EFPIA Participant → assay data, incl. test description, will be provided to Screening Centre
3. Expertise and know-how introduced in review & selection processes (Chemical Library and HTS Project Proposals)
4. Expertise and know-how supporting efficient project realization in Screening Centre; optional provision of assays for hit characterization
## What’s in it for you?

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<th>Category</th>
<th>Benefits</th>
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| **Academia**      | - Access to industry-like small molecule discovery platform to identify quality hit compounds translating cutting-edge projects to drug discovery or target research programmes  
                     - Access to extended expert workbench to realize innovative chemistry proposals => introduce your chemistry to broad pharmaceutical screening activities  
                     - Funding source to translate ideas into value  
                     - Platform to establish early research collaborations  
                     - Unique public-private consortium in the field of early drug discovery => focus on value (IP) generation  
                     - Industry-like discovery engine for projects also outside of pharma focus (e.g. orphan diseases) |
| **SMEs**          | -                                                                                                   |
| **Patients’ Organizations** | -                                                                                                     |
Key deliverables of the full project

- Define processes, incl. internal and external interfaces, and division of work among participants
- Elaborate detailed governance structure
- Provide detailed planning for quantitative project goals, timelines and anticipated budget
- Formulate rules for information management, IP handling, and ownership as drafted in Topic Text

Identify options to compensate for funding gaps

within general framework described in the Topic Text:

http://www.imi.europa.eu
Questions?

• Contact the **IMI Executive Office**
  
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